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TITLE:

Long term expression of recombinant human

factor IX mediated by myoblasts in

mice

AUTHOR(S):

Wang, Hongwei; Yang, Xiaoqing; Bao, Yun; Lu, Daru;

Qiu, Xinfang; Xue, Jinglun

CORPORATE SOURCE:

Department of Hematology, The 2nd Affiliated

Hospital,

Shanxi Medical University, Taiyuan, 030001, Peop.

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The exptl. gene therapy for hemophilia B by using C2C12 cells and C3H

mice

as models was developed. The retroviral **vector** contg. MCK enhancer and hCMV **promoter** was constructed and was used to **transfect** PA317 cells. Then the viral particles were used to **transfect** C2C12 cells. C2C12 cells expressing human **factor IX** (hFIX) protein in vivo was pos. correlated

with the no. of injected cells, and the in vitro expression level of

cell clones. The expression of hFIX could be enhanced and prolonged by immunosuppressive agents. Allogeneic myoblasts transplantation led to immunorejection, but repeated injections at longer internal could improve the survival rate of transplanted cells.

FILE 'MEDLINE, CAPLUS, BIOSIS' ENTERED AT 10:31:01 ON 10 DEC 2001 10623 S FACTOR IX OR BILIRUBIN UDP GLUCURONOSYLTRANSFERASE L1236 S L1 AND VECTOR AND PROMOTER L2 10153 S FACTOR IX L3 L4236 S L1 AND VECTOR AND PROMOTER L51 S L4 AND SERTOLI L6 4 S L4 AND (TRANSFORM OR TRANSFECT) 4 DUP REMOVE L6 (0 DUPLICATES REMOVED) L7L814014 S SERTOLI CELLS L9 27 S L8 AND (TRANSFECT OR TRANSFORM) L10 15 DUP REMOVE L9 (12 DUPLICATES REMOVED)

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L1		10623	S FACTOR IX OR BILIRUBIN UDP GLUCURONOSYLTRANSFERASE
L2		236	S L1 AND VECTOR AND PROMOTER
L3		10153	S FACTOR IX
L4		236	S L1 AND VECTOR AND PROMOTER
L5.		1	S L4 AND SERTOLI
L6		4	S L4 AND (TRANSFORM OR TRANSFECT)
L7		4	DUP REMOVE L6 (0 DUPLICATES REMOVED)
L8		14014	S SERTOLI CELLS
L9		27	S L8 AND (TRANSFECT OR TRANSFORM)
L10		15	DUP REMOVE L9 (12 DUPLICATES REMOVED)